
A treatment for Rett syndrome using glial-restricted neural progenitor cells

Grant Award Details

A treatment for Rett syndrome using glial-restricted neural progenitor cells

Grant Type: Quest - Discovery Stage Research Projects

Grant Number: DISC2-13515

Project Objective: To develop an allogeneic, PSC-derived glial-restricted progenitor cell therapy (GRNPCs) for treating Rett syndrome

Investigator:

Name:	Alysson Muotri
Institution:	University of California, San Diego
Type:	PI

Disease Focus: Autism, Neurological Disorders

Human Stem Cell Use: iPS Cell

Award Value: \$1,402,240

Status: Active

Grant Application Details

Application Title: A treatment for Rett syndrome using glial-restricted neural progenitor cells

Public Abstract:**Research Objective**

We developed a novel glial-restricted neural progenitor cells transplantation strategy as a treatment for Rett syndrome, reverting neuronal alterations caused by genetic mutations.

Impact

There are no disease-modifying therapies for Rett syndrome. Our therapeutic, if successful, will be a first-in-class treatment for this devastating neurological disorder and potentially others.

Major Proposed Activities

- Characterization of the candidate glial-restricted progenitor stem cells production.
- In vitro studies of the cell transplantation in Rett syndrome brain organoids to assess the ability of the cells to revert neuronal alterations at molecular, cellular and circuit levels.
- In vivo efficacy studies upon cell transplantation in the brains of a mice model for Rett syndrome to measure the cellular, physiological, behavioral and survival impact of the treatment.
- Prepare and organize the next steps using large animals to assess immunogenicity, cytotoxicity and off-target effects before moving into clinical trials.

Statement of Benefit to California:

Brain disorders are responsible for more years lost to disability than any other medical condition. Rett syndrome is one of these conditions, affecting Californians independently of race/ethnicity and socioeconomic status. Our therapeutic strategy can be applied to several other neurological conditions, including Parkinson's and Alzheimer's Disease, but also autism spectrum disorders, affecting 1 in every 54 births worldwide, expanding the benefits of the development of this approach.

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